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Hereditary angioedema – Pipeline Insight, 2020

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Abstracts

This report can be delivered to the clients within 72 Hours

DelveInsight's, "Hereditary angioedema – Pipeline Insight, 2020," report provides comprehensive insights about 30+ companies and 30+ pipeline drugs in Hereditary angioedema pipeline landscape. It covers the pipeline drug profiles, including clinical and nonclinical stage products. It also covers the therapeutics assessment by product type, stage, route of administration, and molecule type. It further highlights the inactive pipeline products in this space.

Geography Covered

Global coverage

Hereditary angioedema Understanding

Hereditary angioedema: Overview

Hereditary angioedema is a disorder characterized by recurrent episodes of severe swelling (angioedema). The most common areas of the body to develop swelling are the limbs, face, intestinal tract, and airway. Minor trauma or stress may trigger an attack, but swelling often occurs without a known trigger. Episodes involving the intestinal tract cause severe abdominal pain, nausea, and vomiting. Swelling in the airway can restrict breathing and lead to life-threatening obstruction of the airway. About one-third of people with this condition develop a non-itchy rash called erythema marginatum during an attack.

Symptoms of hereditary angioedema typically begin in childhood and worsen during

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puberty. On average, untreated individuals have an attack every 1 to 2 weeks, and most episodes last for about 3 to 4 days. The frequency and duration of attacks vary greatly among people with hereditary angioedema, even among people in the same family.

There are three types of hereditary angioedema, called types I, II, and III, which can be distinguished by their underlying causes and levels of a protein called C1 inhibitor in the blood. The different types have similar signs and symptoms. Type III was originally thought to occur only in women, b.

Symptoms

The symptoms of Hereditary angioedema includes:

Angioedema

Abdominal pain

Ascites

Facial edema

Intestinal edema

Diagnosis

It is important to note that MOST cases of angioedema or swelling are NOT HAE or C1 Inhibitor Deficiency. Laboratory analysis of blood samples, or genetic samples, are required to establish an HAE diagnosis.

There are three specific blood tests used to confirm Hereditary Angioedema Type I or II.

C1-inhibitor quantitative (antigenic)

C1-inhibitor functional

C4

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Treatment

The medication(s) listed below have been approved by the Food and Drug Administration (FDA) as orphan products for treatment of this condition

C1 esterase inhibitor (human) (Brand name: Cinryze) - Manufactured by ViroPharma Biologics, Inc. was approved June 2018 for routine prophylaxis against angioedema attacks in adults, adolescents and pediatric patients (6 years old and above) with Hereditary Angioedema (HAE).

Icatibant (Brand name: Firazyr) - Manufactured by Shire Orphan Therapies is FDA-approved indication for the treatment of acute attacks of hereditary angioedema in adults 18 years of age and older.

Ecallantide (Brand name: Kalbitor) - Manufactured by Dyax Corp is FDA-approved indication for treatment of acute attacks of hereditary angioedema (HAE) in patients 12 years of age and older

Hereditary angioedema Emerging Drugs Chapters

This segment of the Hereditary angioedema report encloses its detailed analysis of various drugs in different stages of clinical development, including phase III, II, I, preclinical and Discovery. It also helps to understand clinical trial details, expressive pharmacological action, agreements and collaborations, and the latest news and press releases.

Hereditary angioedema Emerging Drugs

BCX7353: BioCryst Pharmaceuticals

Berotralstat (BCX7353) is an oral inhibitor of plasma kallikrein in development for the prevention and treatment of hereditary angioedema (HAE). The US Food and Drug Administration (FDA) has accepted and filed its new drug application (NDA) for the approval of oral, once daily berotralstat (BCX7353). The Prescription Drug User Fee Act (PDUFA) date for the NDA is December 2020.

KVD900: KalVista Pharmaceuticals

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KVD900 is a candidate from our oral portfolio of plasma kallikrein inhibitors. It exhibits high solubility and high permeability and is uniquely suited for on-demand treatment of HAE attacks, with rapid uptake into the plasma and high plasma concentrations. Data in phase II clinical trial for KVD900 is anticipated in the fourth quarter of 2020.

PHA121: Pharvaris

PHA121 is a novel small molecule with drug-like properties. In preclinical studies, PHA121 demonstrates highly potent and selective competitive antagonism of the B2 receptor, and shows rapid and potent activity on oral dosing in a bradykinin-mediated disease model. It is currently in phase I stage of development.

BMN 331: BioMarin Pharmaceutical

BMN 331 is a gene therapy product candidate for HAE. It is currently in preclinical stage of development.

Further product details are provided in the report

Hereditary angioedema: Therapeutic Assessment

This segment of the report provides insights about the different Hereditary angioedema drugs segregated based on following parameters that define the scope of the report, such as:

Major Players in Hereditary angioedema

There are approx. 30+ key companies which are developing the therapies for Hereditary angioedema. The companies which have their Hereditary angioedema drug candidates in the most advanced stage, i.e. NDA include BioCryst Pharmaceuticals and others.

Phases

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DelveInsight's report covers around 30+ products under different phases of clinical development like

Late-stage products (Phase III)

Mid-stage products (Phase II)

Early-stage products (Phase I) along with the details of

Pre-clinical and Discovery stage candidates

Discontinued & Inactive candidates

Route of Administration

Hereditary angioedema pipeline report provides the therapeutic assessment of the pipeline drugs by the Route of Administration. Products have been categorized under various ROAs such as

Infusion

Intradermal

Intramuscular

Intranasal

Intravenous

Oral

Parenteral

Subcutaneous

Topical.

Molecule Type

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Products have been categorized under various Molecule types such as

- Gene therapies
- Small molecule
- Vaccines
- Polymers
- Peptides
- Monoclonal antibodies
- Product Type

Drugs have been categorized under various product types like Mono, Combination and Mono/Combination.

Hereditary angioedema: Pipeline Development Activities

The report provides insights into different therapeutic candidates in phase III, II, I, preclinical and discovery stage. It also analyses Hereditary angioedema therapeutic drugs key players involved in developing key drugs.

Pipeline Development Activities

The report covers the detailed information of collaborations, acquisition and merger, licensing along with a thorough therapeutic assessment of emerging Hereditary angioedema drugs.

Report Highlights

- The companies and academics are working to assess challenges and seek opportunities that could influence Hereditary angioedema R&D. The therapies under development are focused on novel approaches to treat/improve

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Hereditary angioedema.

In February 2020, BioCryst Pharmaceuticals announced that the US Food and Drug Administration (FDA) accepted and filed its new drug application (NDA) for the approval of oral, once daily berotralstat (BCX7353) for the prevention of hereditary angioedema (HAE) attacks.

KVD900 received Fast Track designation from the US FDA, supporting the Company’s belief in the high level of unmet need in HAE and providing a potentially expedited path to drug approval.

BioMarin expect to complete preclinical work with BMN 331 in the year 2020 and in anticipation of a possible clinical trial in early 2021.

Hereditary angioedema Report Insights

Hereditary angioedema Pipeline Analysis

Therapeutic Assessment

Unmet Needs

Impact of Drugs

Hereditary angioedema Report Assessment

Pipeline Product Profiles

Therapeutic Assessment

Pipeline Assessment

Inactive drugs assessment

Unmet Needs

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Key Questions

Current Treatment Scenario and Emerging Therapies:

How many companies are developing Hereditary angioedema drugs?

How many Hereditary angioedema drugs are developed by each company?

How many emerging drugs are in mid-stage, and late-stage of development for the treatment of Hereditary angioedema?

What are the key collaborations (Industry–Industry, Industry–Academia), Mergers and acquisitions, licensing activities related to the Hereditary angioedema therapeutics?

What are the recent trends, drug types and novel technologies developed to overcome the limitation of existing therapies?

What are the clinical studies going on for Hereditary angioedema and their status?

What are the key designations that have been granted to the emerging drugs?

Key Players

BioCryst Pharmaceuticals

KalVista Pharmaceuticals

Pharvaris

BioMarin Pharmaceutical

Ionis Pharmaceuticals, Inc.

Pharming Technologies B.V.

Adverum Biotechnologies

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Intellia Therapeutics

Pharming Group NV

Key Products

BCX7353

KVD900

PHA121

BMN 331

IONIS-PKK-LRx

rhC1INH

ADVM 053

Research Programme: hereditary angioedema therapeutics

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Contents

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List Of Tables

LIST OF TABLES

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List Of Figures

LIST OF FIGURES

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